The expanding world of stem cell modeling of Huntington's disease: creating tools with a promising future.

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Public Summary:
Deconstructing the mechanistic basis of neurodegenerative disorders, such as Huntington's disease (HD), has been a particularly challenging undertaking, relying mostly on post-mortem tissue samples, non-neural cell lines from affected individuals, and model organisms. Two articles recently published in Cell Stem Cell report first the generation and characterization of induced pluripotent stem cell (iPSC)-derived models for HD, and second, the genetic correction of a disease-causing CAG expansion mutation in iPSCs from individuals with HD. Taken together, these two studies provide a framework for the production and validation of iPSC materials for human neurodegenerative disease research and yield crucial tools for investigating future therapies.

Scientific Abstract:
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